

# Controlled Clinical Trials

## DESIGN, METHODS, AND ANALYSIS

Official Journal of the Society for Clinical Trials

Volume 23, Number 1, 2002

### Call for Papers

1

### Original Articles

BRIAN L. WIENS

Choosing an equivalence limit for noninferiority or equivalence studies

2

SUE-JANE WANG, H.M. JAMES HUNG, YI TSONG

Utility and pitfalls of some statistical methods in active controlled clinical trials

15

SHUNSUKE ONO, YASUO KODAMA, TAKU NAGAO, SATOSHI TOYOSHIMA

The quality of conduct in Japanese clinical trials: deficiency is found in GCP inspections

29

TUIJA EKINONEN, SAKARI NIEMINEN, VIRPI SAAREKS, PIRKKO MIETTINEN, VEIJO SAANO, PAULI YLITALO

The quality and characteristics of clinical drug study notifications reviewed by the regulatory agency in Finland

42

### Commentary

WILLIAM C. BLACKWELDER

Showing a treatment is good because it is not bad: when does "noninferiority" imply effectiveness?

52

MASAHIRO TAKEUCHI

The issues to be considered in global drug development

55

### Invited Papers

JAMES D. NEATON

Preface

58

LAURA A. MCNAY, JORGE A. TAVEL, KAREN OSEEKEY, CATHY M. MCDERMOTT, DAVID MOLLERUP, JUDITH D. BEBCHUK, FOR THE ESPRIT GROUP

Regulatory approvals in a large multinational clinical trial: the ESPRIT experience

59

SHEILA A. HEWSON, JULIE WESTON, MARY E. HANNAH

Crossing international boundaries: implications for the Term Breech Trial Data Coordinating Centre

67

LISETTE VAN RAAK, ANNE HILTON, FONS KESSELS, JAN LODDER

Implementing the EGASIS trial, an international multicenter acute intervention trial in stroke

74

MARK D. THORNQUIST, CIM EDELSTEIN, GARY E. GOODMAN, GILBERT S. OMENN

Streamlining IRB review in multisite trials through single-study IRB Cooperative Agreements: experience of the  $\beta$ -Carotene and Retinol Efficacy Trial (CARET)

80

JANET T. HOLBROOK, ROBERT A. WISE, LYNN B. GERALD, FOR THE AMERICAN LUNG ASSOCIATION ASTHMA CLINICAL RESEARCH CENTERS

Drug distribution for a large crossover trial of the safety of inactivated influenza vaccine in asthmatics

87

BARBARA K. MARTIN, CURTIS L. MEINERT, JOHN C.S. BREITNER, FOR THE ADAPT RESEARCH GROUP

Double placebo design in a prevention trial for Alzheimer's disease

93

<b>Letters to the Editor</b>	100
<b>List of Reviewers</b>	103
 <b>Volume 23, Number 2, 2002</b>	
<b>Call for Papers</b>	105
<b>Original Articles</b>	
DAVID DUNN, ABDEL BABIKER, MALCOLM HOOKER, JANET DARBYSHIRE The dangers of inferring treatment effects from observational data: a case study in HIV infection	106
MARY E. PUTT, BERNARD RAVINA Randomized, placebo-controlled, parallel group versus crossover study designs for the study of dementia in Parkinson's disease	111
EMMANUEL LESAFFRE, KRIS BOGAERTS, XIN LI, ERICH BLUHMKI On the variability of covariate adjustment: experience with Koch's method for evaluating the absolute difference in proportions in randomized clinical trials	127
KERSTIN MALMSTROM, IZA PESZEK, AL BOTTO, SUSAN LU, PAUL L. ENRIGHT, THEODORE F. REISS Quality assurance of asthma clinical trials	143
THE DPP RESEARCH GROUP The Diabetes Prevention Program: recruitment methods and results	157
<b>Commentary</b>	
JON F. MERZ The ethics of research on informed consent	172
PETER PEDUZZI, PETER GUARINO, SAM T. DONTA, CHARLES C. ENGEL, JR., DANIEL J. CLAUW, JOHN R. FEUSSNER Making informed consent meaningful: from theory to practice	178
<b>Letters to the Editor</b>	182
<b>Design papers</b>	
PETER PEDUZZI, PETER GUARINO, SAM T. DONTA, CHARLES C. ENGEL, JR., DANIEL J. CLAUW, JOHN R. FEUSSNER Research on informed consent: investigator-developed versus focus group-developed consent documents, a VA cooperative study	184
SEAN EMERY, DONALD I. ABRAMS, DAVID A. COOPER, JANET H. DARBYSHIRE, H. CLIFFORD LANE, JENS D. LUNDGREN, JAMES D. NEATON, AND THE ESPRIT STUDY GROUP The Evaluation of Subcutaneous Proleukin® (interleukin-2) in a Randomized International Trial: rationale, design, and methods of ESPRIT	198
 <b>Volume 23, Number 2S, 2002</b>	
<b>Supplement</b>	
Letter from the President	1S
Calendar of Program	5S
Abstracts: Twenty-third Annual Meeting of the Society for Clinical Trials	37S
Author/Speaker Index	95S

**Volume 23, Number 3, 2002**

<b>Call for Papers</b>	221
<b>Original Articles</b>	
PHILIP W. LAVORI, HEIDI KRAUSE-STEINRAUF, MARY BROPHY, JOEL BUxBAUM, JENNIFER COCKROFT, DAVID R. COX, LOUIS FIORE, HENRY T. GREELY, HARRY GREENBERG, EDWARD W. HOLMES, LORENE M. NELSON, JEREMY SUGARMAN Principles, organization, and operation of a DNA bank for clinical trials: a Department of Veterans Affairs cooperative study	222
THOMAS M. BRAUN The bivariate continual reassessment method: extending the CRM to phase I trials of two competing outcomes	240
SALLY GALBRAITH, IAN C. MARSCHNER Guidelines for the design of clinical trials with longitudinal outcomes	257
SCOTT D. HALPERN Prospective preference assessment: a method to enhance the ethics and efficiency of randomized controlled trials	274
LINDA K. LARKEY, LISA K. STATEN, CHERYL RITENBAUGH, RENÉE A. HALL, DAVID B. BULLER, TAMSEN BASSFORD, BARBARA REMPFER ALTIMARI Recruitment of Hispanic women to the Women's Health Initiative: the case of <i>Embajadoras</i> in Arizona	289
<b>Book Review</b>	299
<b>Design papers</b>	
TIMOTHY A. DEROUEN, BRIAN G. LEROUX, MICHAEL D. MARTIN, BRENDA D. TOWNES, JAMES S. WOODS, JORGE LEITÃO, ALEXANDRE CASTRO-CALDAS, NORMAN BRAVEMAN Issues in design and analysis of a randomized clinical trial to assess the safety of dental amalgam restorations in children	301
J. ATHENE LANE, RICHARD F. HARVEY, LIAM J. MURRAY, IAN M. HARVEY, JENNY L. DONOVAN, PRAKASH NAIR, MATTHIAS EGGER A placebo-controlled randomized trial of eradication of <i>Helicobacter pylori</i> in the general population: study design and response rates of the Bristol Helicobacter Project	321
JOSEPH F. COLLINS, SAM T. DONTA, CHARLES C. ENGEL JR., JOEL B. BASEMAN LISA L. DEVER, THOMAS TAYLOR, KATHY D. BOARDMAN, SUZANNE E. MARTIN, ANNETTE L. WISEMAN, JOHN R. FEUSSNER FOR THE VA #475 GWVI ANTIBIOTIC TREATMENT TRIAL GROUP The Antibiotic Treatment Trial of Gulf War Veterans' Illnesses: issues, design, screening, and baseline characteristics	333

**Volume 23, Number 4, 2002**

<b>Original articles</b>	
BORIS FREIDLIN, EDWARD L. KORN A comment on futility monitoring	355
KATHERINE S. PANAGEAS, ALEX SMITH, MITHAT GÖNEN, PAUL B. CHAPMAN An optimal two-stage phase II design utilizing complete and partial response information separately	367

P.J. DEVEREAUX, BRADEN J. MANNS, WILLIAM A. GHALI, HUDE QUAN, GORDON H. GUYATT	
The reporting of methodological factors in randomized controlled trials and the association with a journal policy to promote adherence to the Consolidated Standards of Reporting Trials (CONSORT) checklist	380
BARBARA H. PERRY, ALLAN R. SAMPSON, BARRY H. SCHWAB, M. REZAUL KARIM, JANICE M. SMIELL	
A meta-analytic approach to an integrated summary of efficacy: a case study of bevacizumab gel	389
LIAM SMEETH, EDMOND SIU-WOON NG	
Intraclass correlation coefficients for cluster randomized trials in primary care: data from the MRC Trial of the Assessment and Management of Older People in the Community	409
<b>Letters to the editor</b>	422
<b>Book review</b>	429
<b>Design papers</b>	
ZIDING FENG, BETI THOMPSON	
Some design issues in a community intervention trial	431
THE WGET RESEARCH GROUP	
Design of the Wegener's Granulomatosis Etanercept Trial (WGET)	450
 <b>Volume 23, Number 5, 2002</b>	
<b>Original articles</b>	
ANDREAS KOOP, RALPH MÖSGES	
The use of handheld computers in clinical trials	469
QILONG YI, TONY PANZARELLA	
Estimating sample size for tests on trends across repeated measurements with missing data based on the interaction term in a mixed model	481
HUNG-MO LIN, ROBERT H. LYLES, JOHN M. WILLIAMSON	
Bias in a placebo-controlled study due to mismeasurement of disease status and the regression effect	497
VANCE W. BERGER	
Improving the information content of categorical clinical trial endpoints	502
SHEIN-CHUNG CHOW, JUN SHAO	
A note on statistical methods for assessing therapeutic equivalence	515
ELLEN GRAHAM RENFROE, GEOFF HEYWOOD, LYNNE FOREMAN, ELEANOR SCHRON, JUDY POWELL, CHRISTINA BAESSLER, DEBORAH WARWICK, MARY MORRIS, ALFRED HALLSTROM, FOR THE AVID COORDINATORS AND INVESTIGATORS	
The end-of-study patient survey: methods influencing response rate in the AVID Trial	521
<b>Letter to the editor</b>	534
<b>Book reviews</b>	536

## **Design papers**

MAURICIO L. BARRETO, LAURA C. RODRIGUES, SERGIO S. CUNHA, SUSAN PEREIRA, MIGUEL A. HIJJAR, MARIA YURY ICHIHARA, SILVANA C. DE BRITO, INES DOURADO Design of the Brazilian BCG-REVAC trial against tuberculosis: a large, simple randomized community trial to evaluate the impact on tuberculosis of BCG revaccination at school age	540
LAWRENCE S. PHILLIPS, VICKI S. HERTZBERG, CURTISS B. COOK, IMAD M. EL-KEBBI, DANIEL L. GALLINA, DAVID C. ZIEMER, CHRISTOPHER D. MILLER, JOYCE P. DOYLE, CATHERINE S. BARNES, WRENN SLOCUM, ROBERT H. LYLES, RISA P. HAYES, DENNIS N. THOMPSON, DAVID J. BALLARD, WILLIAM M. MCCLELLAN, WILLIAM T. BRANCH, JR. The Improving Primary Care of African Americans with Diabetes (IPCAAD) project: rationale and design	554
VALERIE L. DURKALSKI, YUKO Y. PALESCH, BENOIT C. PINEAU, DAVID J. VINING, PETER B. COTTON The Virtual Colonoscopy Study: a large multicenter clinical trial designed to compare two diagnostic screening procedures	570
ANDREA L. DUNN, MADHUKAR H. TRIVEDI, JAMES B. KAMPERT, CAMILLIA G. CLARK, HEATHER O. CHAMBLISS The DOSE study: a clinical trial to examine efficacy and dose response of exercise as treatment for depression	584
<b>Announcement</b>	<b>605</b>

## **Volume 23, Number 6, 2002**

### **Contents**

#### **Original articles**

GEERT MOLENBERGHHS, MARC BUYSE, HELENA GEYS, DIDIER RENARD, TOMASZ BURZYKOWSKI, ARIEL ALONSO Statistical challenges in the evaluation of surrogate endpoints in randomized trials	607
JEREMY M.G. TAYLOR, YAN WANG Surrogate markers and joint models for longitudinal and survival data	626
MICHAEL P. McDERMOTT, W.J. HALL, DAVID OAKES, SHIRLEY EBERLY Design and analysis of two-period studies of potentially disease-modifying treatments	635
RAPHAËL PORCHER, VINCENT LÉVY, SYLVIE CHEVRET Sample size correction for treatment crossovers in randomized clinical trials with a survival endpoint	650
NEIL W. SCOTT, GLADYS C. MCPHERSON, CRAIG R. RAMSAY, MARION K. CAMPBELL The method of minimization for allocation to clinical trials: a review	662
DONNA K. PAULER, KATHRYN B. GOWER, PHYLLIS J. GOODMAN, JOHN J. CROWLEY, IAN M. THOMPSON Biomarker-based methods for determining noncompliance in a prevention trial	675
HOWARD D. SESSO, J. MICHAEL GAZIANO, MARTIN VANDENBURGH, CHARLES H. HENNEKENS, ROBERT J. GLYNN, JULIE E. BURING Comparison of baseline characteristics and mortality experience of participants and nonparticipants in a randomized clinical trial: the Physicians' Health Study	686

**Commentary****MICHAEL D. HUGHES**

Evaluating surrogate endpoints

703

**Design papers**

JUDITH HSIA, EDWIN L. ALDERMAN, JOEL I. VERTER, WILLIAM J. ROGERS,  
PAUL THOMPSON, BARBARA V. HOWARD, FREDERICK R. COBB,  
PAMELA OUYANG, JEAN CLAUDE TARDIF, LYALL HIGGINSON, VERA BITTNER,  
IVAN BAROFSKY, MICHAEL STEFFES, DAVID J. GORDON, MICHAEL PROSCHAN,  
NAJI YOUNES, DAVID WATERS

Women's Angiographic Vitamin and Estrogen trial: design and methods

708

JOHN P. PIERCE, SUSAN FAERBER, FRED A. WRIGHT, CHERYL L. ROCK,  
VICKY NEWMAN, SHIRLEY W. FLATT, SHEILA KEALEY, VICKY E. JONES,  
BETTE J. CAAN, ELLEN B. GOLD, MARY HAAN, KATHRYN A. HOLLENBACH,  
LOVELL JONES, JAMES R. MARSHALL, CHERYL RITENBAUGH,  
MARCIA L. STEFANICK, CYNTHIA THOMSON, LINDA WASSERMAN,  
LOKI NATARAJAN, RONALD G. THOMAS, ELIZABETH A. GILPIN, FOR THE WOMEN'S  
HEALTHY EATING AND LIVING (WHEL) STUDY GROUP

A randomized trial of the effect of a plant-based dietary pattern on additional  
breast cancer events and survival: the Women's Healthy Eating and Living  
(WHEL) Study

728

JERRY AVORN, JOSH BENNER, IAN FORD, DAVID A. GANZ, ALLAN GAW,  
ROBERT J. GLYNN, JOSEPH JACKSON, A. MARGOT LAGAAY,  
SEBASTIAN SCHNEEWEISS, THOMAS WALLEY, PHILIP S. WANG, ON BEHALF OF  
THE OUTCOMES RESEARCH WORKING GROUP AND THE PROSPER STUDY GROUP

Measuring the cost-effectiveness of lipid-lowering drugs in the elderly: the  
outcomes research and economic analysis components of the PROSPER trial

757

**Errata**

774

**Volume 23 Author Index**

775

**Volume 23 Subject Index**

779

**Volume Contents**

789